Population: the entire collection of individuals/objects of interest

<u>Sample:</u> a subset of the population

Types of Studies

• Experiment: researcher manipulates 1 or more independent variables (Factors)

Treatments are the levels for the factors (or combinations thereof)

Experimental Unit (EU)

- the unit to which a treatment is randomly assigned - An EU constitutes one <u>replication</u> of the experiment

Measurement Unit

- the unit on which a measurement is taken
- An EU constitutes one replication of the experiment
- <u>Quasi-Experiment:</u> treatments not randomly assigned to EUs
- <u>Observational:</u> no variables manipulated by researcher
 - experiment may not be feasible or ethical
- <u>Survey:</u> voluntary response
- Causal inference can be made from an experiment

Association relationships can be inferred from an observational study

Experimental Error - Variation among identically treated EUs

- Natural variation among EUs
- Measurement variability
- Variation in treatment conditions
- Extraneous factors (nuisance/lurking variables)
- Interaction of treatments and EUs

Control Treatments - A benchmark for comparing experimental treatments

- No treatment
- Placebo
- Standard practice

3 Principles of Designed Experiments

- 1) <u>Blocking</u> to reduce experimental error
- 2) <u>Randomization</u> to reduce hidden bias
- 3) <u>Replication</u> on an large number of subjects

Key Ingredients to identify: A hypothesis

A hypothesis Dependent variable(s) Experimental conditions Nuisance variables # of EUs Assignment mechanism

Blocking - Grouping of EUs into similar classes

- Common Criteria for blocking
 - o Location
 - o Characteristics (age, weight, sex, ...)
 - o Time

Randomization - Random assignment of treatments to EUs

- Independent observations needed for valid estimates of experimental error
- Randomization simulates the effect of independence
 - o Allows the assumption of independence & normal distribution

Replication

- Demonstrates reproducibility
- Allows for increased precision in estimating treatment effects

Surveys

- Administered to a sample from the population to gather information about the entire pop.
- Possible Problems:

- non-response, incomplete recall, leading questions, unclear questions \rightarrow <u>Bias</u> – when a study systematically favors certain outcomes

Sampling Designs for Surveys

- <u>Simple Random Sample (SRS):</u>
 - A method of slecting n individuals from a pop. so that each is equally likely to be selected
- <u>Stratified Random Sample:</u>
 - Divide the population into groups of similar individuals (strata)
 - Take a Simple Random Sample from within each stratum
- Cluster Sampling:
 - Divide the population into groups of similar individuals (clusters)
 - Select a subset of clusters, and sample all individuals in the selected clusters
- Systematic Sampling:
 - Select every \boldsymbol{k}^{th} individual from the population
 - May be more convenient, but is less efficient than other methods
 - Potential for bias is higher than for other methods

The National Health Interview Survey (NHIS)

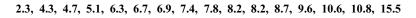
- Conducted by the Census Bureau for the National Center for Health Statistics (NCHS) <u>Uses:</u>
 - o to help set public policy
 - o to track progress of national health objectives
 - $\circ\;\;$ to aid in research (in conjunction with the Medical Expenditure Panel Survey)
- <u>Target Population</u>: U.S. resident, civilian, non-institutionalized persons
- <u>Sampling Frame</u>: geographic areas defined in 3 stages
- <u>Sampling Design</u>: stratified multi-stage probability sample
- <u>Components</u>: Core Survey and usually 4 Supplements

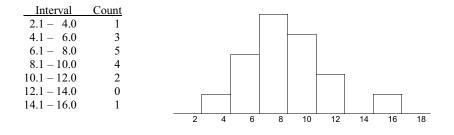
1993 Sample:

- 43,007 households interviewed \rightarrow 109,671 persons
- Non-interview rate: 4.4%

Histogram

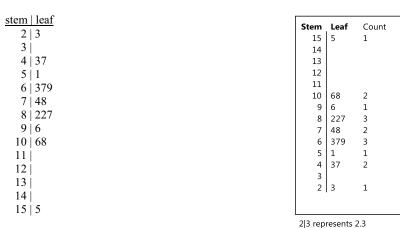
- 1. Divide the range of values into equal length intervals
- 2. Count the # of observations in each interval
- 3. Plot adjacent rectangles with height equal to the count (or %) in each interval
- <u>Example</u>: Enzyme Data (concentrations sorted from lowest to highest)





Stem & Leaf Plot

- The <u>stem</u> consists of all but the right most digit(s)
- The <u>leaf</u> consists of the last digit(s)



(The decimal point is at the |)

Stem & Leaf Plot

- A histogram-like plot that allows you to recover the actual data
- The <u>stem</u> consists of all but the right most digit(s)
- The <u>leaf</u> consists of the last digit(s)

Procedure:

- 1. Write the stems in a column in increasing order
- 2. Use a vertical line to represent the decimal point
- 3. Write the leaves in increasing order next to the corresponding stem

Measures of Center

• Mean: ordinary average

$$\overline{x} = \frac{x_1 + x_2 + \dots + x_n}{n} = \frac{\sum_i x_i}{n}$$

- Median (M): the "middle value" of the ordered data
 - $\frac{1}{2}$ of the values are larger, $\frac{1}{2}$ of the values are smaller
 - Procedure: Order the sample from smallest to largest
 - If *n* is odd, *M* is the middle value
 - If *n* is even, *M* is the average of the two middle values
 - The location of **M** among the ranked values is (n+1)/2

Example Data: 5, 4, 2, 6, 3

Measures of Spread

- Range: the difference between the largest and smallest values
- Quartiles: break a distribution into 4 intervals

 - ¹/₄ of the observations are less than the 1st Quartile (Q1 = 25th Percentile)
 ¹/₂ of the observations are less than the 2nd Quartile (Q2 = 50th Percentile)
 - $\frac{3}{4}$ of the observations are less than the 3^{rd} Quartile (Q3 = 75th Percentile)

Inter Quartile Range (IQR): the difference between the 3rd and 1st Quartiles

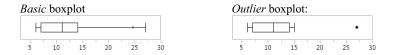
- IOR = O3 O1
- Measures the spread of the middle half of the data
- 5 Number Summary: Minimum, Q1, Median, Q3, Maximum
- Variance and Standard Deviation:

Ex Data: 7 8 12 12 14 15 16 28 7 9 11

Example Data: 7 7 8 9 11 12 12 14 15 16 28

Boxplot: A graphical representation of a 5 number summary (developed by John Tukey)

- A central box at Q1 and Q3 with a line at the Median
- 'wiskers' extending to ...
 - the Min and Max (Basic or "Skeletal" boxplot), OR
 - the lower and upper adjacent values (*Outlier* boxplot) [outliers indicated by *]



- Lower Inner Fence: $LIF = O_1 - 1.5 * IOR$
- Upper Inner Fence: $UIF = Q_3 + 1.5 * IOR$
- **Outlier**: any observed value <*LIF* or >*UIF*
- Lower Adjacent Value (LAV): the smallest non-outlier
- Upper Adjacent Value (UAV): the largest non-outlier